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(FILE 'HOME' ENTERED AT 18:40:17 ON 17 MAY 2007)

FILE 'MEDLINE, CAPLUS, BIOSIS, SCISEARCH, LIFESCI' ENTERED AT 18:40:34 ON 17 MAY 2007

- 11835 S LYSOSOMAL(4A) (DISEASE OR DISORDER)
- L2 985 S (TREAT? OR INHIBIT? OR AMELIORAT? OR DECREAS? OR REDUC?) (8A)L
- L3 147288 S GLUCOCEREBROSIDASE OR CERAMIDASE OR GALACTOCEREBROSIDASE OR S
- L4 212 S L2 AND L3
- L5 121 DUP REM L4 (91 DUPLICATES REMOVED)
- L6 10097 S (GENE OR DNA OR CDNA OR POLYNUCLEOTIDE OR NUCLEIC (W) ACID) (8A)
- L7 36 S L2 AND L6
  - 20 DUP REM L7 (16 DUPLICATES REMOVED)
- L9 7112 S L3 (5A) (PROTEIN OR POLYPEPTIDE)
- L10 16 S L2 AND L9
- L11 11 DUP REM L10 (5 DUPLICATES REMOVED)
- => d au ti so pi 1-20 18
- L8 ANSWER 1 OF 20 CAPLUS COPYRIGHT 2007 ACS on STN
- AU Cardone, Monica; Polito, Vinicia Assunta; Pepe, Stefano; Mann, Linda; D'Azzo, Alessandra; Auricchio, Alberto; Ballabio, Andrea; Cosma, Maria Pia
- TI Correction of Hunter syndrome in the MPSII mouse model by AAV2/8-mediated gene delivery
- SO Human Molecular Genetics (2006), 15(7), 1225-1236 CODEN: HMGEE5; ISSN: 0964-6906
- L8 ANSWER 2 OF 20 CAPLUS COPYRIGHT 2007 ACS on STN
- IN Erwin, Robert L.; Grill, Laurence K.; Pogue, Gregory P.; Turpen, Thomas H.; Kumagai, Monto H.
- TI Manufacture of mammalian lysosomal enzymes for enzyme replacement therapy in plants using viral expression vectors
- SO U.S., 59 pp., Cont.-in-part of U.S. Ser. No. 316,572, abandoned.

	CODEN: USXXAM PATENT NO.					-	-											
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	ES	2171	398			T3		2002	0916		ES 1	1992-	9164	41		1	9920	731
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	US	5589	367			A		1996	1231		US 1	994-	1842	37		1:	9940	119
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Kim Eun Young; Hong Young Bin; Lai Zhennan; Cho Youl-Hee; Brady Roscoe O;
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- L8 ANSWER 3 OF 20
- ΑU Jung Sung-Chul
- TI Long-term expression of the human glucocerebrosidase gene in vivo after transplantation of bone-marrow-derived cells transformed with a lentivirus vector.
- SO The journal of gene medicine, (2005 Jul) Vol. 7, No. 7, pp. 878-87. Journal code: 9815764. ISSN: 1099-498X.
- L8 ANSWER 4 OF 20 CAPLUS COPYRIGHT 2007 ACS on STN
- IN Passini, Marco A.; Stewart, Greg
- ΤI Gene therapy for neurometabolic disorders
- SO PCT Int. Appl., 50 pp.

	CODEN:	PIXXD2											
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		TJ, TM,											
	RW:	BW, GH,											
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- L8 ANSWER 5 OF 20 CAPLUS COPYRIGHT 2007 ACS on STN
- IN Fan, Jian-Qiang
- ΤI Combination therapy for treating protein deficiencies
- SO PCT Int. Appl., 40 pp.

CODEN: PIXXD2

	PA:	<b>FENT</b>	NO.			KIN	D	DATE			APPL	ICAT	ION 1	NO.		D	ATE	
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ΡI	WO	2004	0744	50		A2		2004	0902		WO 2	004-1	US49	09		2	0040	218
	WO	2004	0744	50		<b>A8</b>		2004	1104									
	WO 2004074450				<b>A3</b>		2005	0825										
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IN 2005DN04230
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                     Α
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- L8 ANSWER 6 OF 20 CAPLUS COPYRIGHT 2007 ACS on STN
- IN Cheng, Seng H.; Meeker, David
- TI Combined enzyme replacement, gene therapy and small molecule therapy for lysosomal storage diseases
- SO U.S. Pat. Appl. Publ., 35 pp., Cont.-in-part of U.S. Ser. No. 884,526. CODEN: USXXCO

	PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
ΡI	US 2004204379	<b>A1</b>	20041014	US 2004-758773	20040116
	US 2002095135	A1	20020718	US 2001-884526	20010619

- L8 ANSWER 7 OF 20 MEDLINE on STN
- AU Van den Hout Johanna M P; Kamphoven Joep H J; Winkel Leon P F; Arts Willem F M; De Klerk Johannes B C; Loonen M Christa B; Vulto Arnold G; Cromme-Dijkhuis Adri; Weisglas-Kuperus Nynke; Hop Wim; Van Hirtum Hans; Van Diggelen Otto P; Boer Marijke; Kroos Marian A; Van Doorn Pieter A; Van der Voort Edwin; Sibbles Barbara; Van Corven Emiel J J M; Brakenhoff Just P J; Van Hove Johan; Smeitink Jan A M; de Jong Gerard; Reuser Arnold J J; Van der Ploeg Ans T
- TI Long-term intravenous treatment of Pompe disease with recombinant human alpha-glucosidase from milk.
- SO Pediatrics, (2004 May) Vol. 113, No. 5, pp. e448-57. Journal code: 0376422. E-ISSN: 1098-4275.
- L8 ANSWER 8 OF 20 SCISEARCH COPYRIGHT (c) 2007 The Thomson Corporation on STN
- AU Van den Hout J M P; Kamphoven J H J; Winkel L P F; Arts W F M; De Klerk J B C; Loonen M C B; Vulto A G; Cromme-Dijkhuis A; Weisglas-Kuperus N; Hop W; Van Hirtum H; Van Diggelen O P; Boer M; Kroos M A; Van Doorn P A; Van der Voort E; Sibbles B; Van Corven E J J M; Brakenhoff J P J; Van Hove J; Smeitink J A M; de Jong G; Reuser A J J; Van der Ploeg A T (Reprint)
- TI Long term intravenous treatment of Pompe disease with recombinant human alpha-glucosidase from milk
- SO PEDIATRICS, (MAY 2004) Vol. 113, No. 5, pp. E448-E457. ISSN: 0031-4005.
- L8 ANSWER 9 OF 20 MEDLINE on STN DUPLICATE 2
- AU Eto Y; Shen J-S; Meng X-L; Ohashi T
- TI Treatment of lysosomal storage disorders: cell therapy and gene therapy.
- SO Journal of inherited metabolic disease, (2004) Vol. 27, No. 3, pp. 411-5.
  Ref: 4
  Journal code: 7910918. ISSN: 0141-8955.
- L8 ANSWER 10 OF 20 CAPLUS COPYRIGHT 2007 ACS on STN
- IN Garger, Stephen J.; Turpen, Thomas H.; Kumagai, Monto H.
- TI Production of human glucocerebrosidase and  $\alpha$  galactosidase in

transgenic tobacco plants and their potential use in enzyme replacement therapy and treatment of lysosomal storage diseases

U.S. Pat. Appl. Publ., 88 pp., Cont.-in-part of U.S. Ser. No. 626,127. so

	PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
ΡI	US 2002088024	A1	20020704	US 2001-993059	20011113
	US 6887696	B2	20050503		
	AU 9918610	A	19990506	AU 1999-18610	19990305
	AU 741443	B2	20011129		
	US 6846968	B1	20050125	US 2000-626127	20000726
	US 2003106095	A1	20030605	US 2002-103327	20020320
	US 6890748	B2	20050510		
	US 2004016021	<b>A1</b>	20040122	US 2003-602219	20030623
	US 2004023281	A1	20040205	US 2003-602220	20030623
	US 2004234516	<b>A1</b>	20041125	US 2004-851388	20040521
	US 2005125859	A1	20050609	US 2004-984389	20041108

- ANSWER 11 OF 20 CAPLUS COPYRIGHT 2007 ACS on STN L8
- Ziegler, Robin; Cheng, Seng; Marshall, John; Goldberg, Mark IN
- TI Methods for treatment of lysosomal storage diseases
- PCT Int. Appl., 25 pp. so

CODEN: PIXXD2

	PATENT NO.	KIND DATE	APPLICATION NO.	DATE
PI	WO 2001060377	A2 20010823	WO 2001-US3875	20010206
	WO 2001060377	A3 20020418		
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	US 2001031741	A1 20011018	US 2001-777743	20010206
	US 2004110709	A1 20040610	US 2003-632302	20030801

- ANSWER 12 OF 20 CAPLUS COPYRIGHT 2007 ACS on STN L8
- IN Podsakoff, Gregory; Watson, Gordon; Couto, Linda B.; Yang, Bin
- Recombinant adeno-associated virus vectors for the treatment of ΤI lysosomal storage disorders and methods of delivering the vectors to a mammalian subject
- SO PCT Int. Appl., 97 pp.

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PI	WO	2001	03660	03		A2		2001	0525	1	WO 2	000-1	US31	688		20	0001	117
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	US 2003219414			A1		2003	1127	1	US 2	003-	4211	75		20	00304	422		
	US 2006104954				A1		2006	0518	1	US 2	006-	3299	41		20	0060	111	

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- Davidson, Beverly L.; Jolly, Douglas J.; Sauter, Sybille L.; Ghodsi, Adbi; IN Stein, Colleen S.; Derksen, Todd A.; Dubensky, Thomas W., Jr.
- Recombinant gene delivery vectors for treating or preventing lysosomal storage disorders

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SO
     PCT Int. Appl., 52 pp.
     CODEN: PIXXD2
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     PATENT NO.
                        KIND
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A1 20020306 EP 2000-937832
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    ANSWER 14 OF 20 CAPLUS COPYRIGHT 2007 ACS on STN
    Amalfitano, Andrea; Chen, Yuan Tsong; Hu, Huimin
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L8
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    Amalfitano, A.; McVie-Wylie, A. J.; Hu, H.; Dawson, T. L.; Raben, N.;
ΑŲ
    Plotz, P.; Chen, Y. T.
    Systemic correction of the muscle disorder glycogen storage disease type
TI
    II after hepatic targeting of a modified adenovirus vector encoding human
    acid-α-glucosidase
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SO Proceedings of the National Academy of Sciences of the United States of America (1999), 96(16), 8861-8866

CODEN: PNASA6; ISSN: 0027-8424

- AU Bijvoet A G; Kroos M A; Pieper F R; de Boer H A; Reuser A J; van der Ploeg A T; Verbeet M P
- TI Expression of cDNA-encoded human acid alpha-glucosidase in milk of transgenic mice.
- SO Biochimica et biophysica acta, (1996 Aug 14) Vol. 1308, No. 2, pp. 93-6. Journal code: 0217513. ISSN: 0006-3002.
- L8 ANSWER 17 OF 20 MEDLINE on STN DUPLICATE 4
- AU Schmidt B; Selmer T; Ingendoh A; von Figura K
- TI A novel amino acid modification in sulfatases that is defective in multiple sulfatase deficiency.
- SO Cell, (1995 Jul 28) Vol. 82, No. 2, pp. 271-8. Journal code: 0413066. ISSN: 0092-8674.
- L8 ANSWER 18 OF 20 CAPLUS COPYRIGHT 2007 ACS on STN
- AU Litjens, Tom; Morris, C. Phillip; Gibson, Gary J.; Beckmann, Kerri R.; Hopwood, John J.
- TI Human N-acetylgalactosamine-4-sulphatase: protein maturation and isolation of genomic clones
- SO Biochemistry International (1991), 24(2), 209-15 CODEN: BIINDF; ISSN: 0158-5231
- L8 ANSWER 19 OF 20 CAPLUS COPYRIGHT 2007 ACS on STN
- AU Louie, Eileen; Rafi, Mohammad A.; Wenger, David A.
- TI Leukocyte sonicates as a source for both enzyme assay and DNA amplification for mutational analysis of certain lysosomal disorders
- SO Clinica Chimica Acta (1991), 199(1), 7-15 CODEN: CCATAR; ISSN: 0009-8981
- L8 ANSWER 20 OF 20 MEDLINE on STN DUPLICATE 5
- AU Waheed A; Hasilik A; von Figura K
- TI Enhanced breakdown of arylsulfatase A in multiple sulfatase deficiency.
- SO European journal of biochemistry / FEBS, (1982 Apr 1) Vol. 123, No. 2, pp. 317-21.
  - Journal code: 0107600. ISSN: 0014-2956.

## => d au ti so pi 1-11 l11

- L11 ANSWER 1 OF 11 CAPLUS COPYRIGHT 2007 ACS on STN
- IN Galindo, Silvia Muro; Muzykantov, Vlamidir R.; Schuchman, Edward Howard
- TI Targeted protein replacement for the treatment of lysosomal storage disorders
- SO PCT Int. Appl., 61 pp.

CODEN: PIXXD2

	PATENT NO.			KIN	D	DATE		1	APPL	ICAT	ION :	NO.		D	ATE			
ΡI	WO	2006	 0075	60		A2	-	2006	 0119	,	WO 2	 005-1	 US23	 529		20	0050	 701
	WO	2006	0075	60		A3		2006	1019									
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			SL,	SM,	SY,	TJ,	TM,	TN,	TR,	TT,	TZ,	UA,	UG,	US,	UZ,	VC,	VN,	ΥU,
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			KG,	ΚZ,	MD,	RU,	ТJ,	TM										

- L11 ANSWER 2 OF 11 CAPLUS COPYRIGHT 2007 ACS on STN
- IN Zankel, Todd; Starr, Christopher M.; Gabathuler, Reinhard

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lipoprotein receptor-related proteins for the treatment of CNS
     and lysosomal storage diseases
so
     PCT Int. Appl., 192 pp.
     CODEN: PIXXD2
     PATENT NO.
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     WO 2005002515
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                          A2
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                                                                      20040617
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                          A3
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                                                                      20030620
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                           A1
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     EP 1638605
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L11
     ANSWER 3 OF 11 CAPLUS COPYRIGHT 2007 ACS on STN
ΑU
     Vaags, Andrea K.; Campbell, Tessa N.; Choy, Francis Y. M.
TI
     HIV TAT variants differentially influence the production of
     glucocerebrosidase in Sf9 cells
SO
     GMR, Genetics and Molecular Research (2005), 4(3), 491-495
     CODEN: GGMRBI; ISSN: 1676-5680
     URL: http://www.funpecrp.com.br/gmr/year2005/vol3-4/pdf/gmr0168.pdf
     ANSWER 4 OF 11 CAPLUS COPYRIGHT 2007 ACS on STN
L11
     Shaaltiel, Yoseph; Baum, Gideon; Bartfeld, Daniel
ΙN
     Production of high mannose proteins in plant culture
TI
so
     PCT Int. Appl., 102 pp.
     CODEN: PIXXD2
     PATENT NO.
                          KIND
                                 DATE
                                            APPLICATION NO.
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PΙ
     WO 2004096978
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                           A1
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     BR 2004010520
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                           Т
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     CN 1875111
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                                 20061206
                                              CN 2004-80017916
                                                                      20040224
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Delivery of therapeutic compounds to the brain and other tissues through

TI

US 2005-554387 20051025 US 2006204487 A1 20060914

L11 ANSWER 5 OF 11 CAPLUS COPYRIGHT 2007 ACS on STN IN Fan, Jian-Qiang Combination therapy for treating protein deficiencies ΤI

PCT Int. Appl., 40 pp. SO CODEN: PIXXD2

	PATENT	NO.	KIN			AP	PLICAT	ION 1	. 01		D	ATE	
PI		1074450 1074450	A2	2004		_	2004-	US490	9		20	0040	218
	WO 2004	074450	A3	2005	0825								
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		304			0902	CA	2004-	25163	304		20	040	218
	EP 1594	1514	A2	2005	1116	EP	2004-	71242	28		20	040	218
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		834			_		2004-	80004	4467		20	040	218
		5517980					2006-					040	
	IN 2005	DN04230	Α	2007	0427	IN	2005-	DN423	30		20	0050	919

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- Fusion proteins comprising immunoglobulin and target antigen with reduced TI T cell epitope and immunogenicity for therapeutic use

SO PCT Int. Appl., 92 pp.

50	CODEN:	PIXXD2	JZ pp.							
	PATENT			ID DATE			TION NO.		DATE	
PI	WO 2002	066514	A2	20020	0829				20020	218
	WO 2002	066514	A3	20030	213					
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		PL, PT,	RO, RU,	SD, SE,	SG, SI,	SK, SL,	TJ, TM,	TN,	TR, TT,	TZ,
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				20031						
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	HU 2003	03171	A2	20031	1229	HU 2003-	3171		20020	218
		007267			210	BR 2002-	7267		20020	218
		768					805158			218
		532620			L028 .	JP 2002-	566227		20020	218
		082039				US 2003-	468370		20030	819
				20070						
	ZA 2003	007323	Α	20040	706	ZA 2003-	7323		20030	918

- L11 ANSWER 7 OF 11 CAPLUS COPYRIGHT 2007 ACS on STN
- IN Garger, Stephen J.; Turpen, Thomas H.; Kumagai, Monto H.
- TI Production of human glucocerebrosidase and  $\alpha$  galactosidase in transgenic tobacco plants and their potential use in enzyme replacement therapy and treatment of lysosomal storage diseases
- SO U.S. Pat. Appl. Publ., 88 pp., Cont.-in-part of U.S. Ser. No. 626,127. CODEN: USXXCO

	PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI	US 2002088024	A1	20020704	US 2001-993059	20011113
	US 6887696	B2	20050503		
	AU 9918610	Α	19990506	AU 1999-18610	19990305
	AU 741443	B2	20011129		
	US 6846968	B1	20050125	US 2000-626127	20000726
	US 2003106095	A1	20030605	US 2002-103327	20020320
	US 6890748	B2	20050510		
	US 2004016021	A1	20040122	US 2003-602219	20030623
	US 2004023281	A1	20040205	US 2003-602220	20030623
	US 2004234516	A1	20041125	US 2004-851388	20040521
	US 2005125859	A1	20050609	US 2004-984389	20041108

- L11 ANSWER 8 OF 11 CAPLUS COPYRIGHT 2007 ACS on STN
- IN D'azzo, Alessandra; Bonten, Erik Jacobus
- TI Targeting proteins to cells expressing mannose receptors via expression in insect cells
- SO U.S. Pat. Appl. Publ., 10 pp.

CODEN: USXXCO PATENT NO. DATE APPLICATION NO. KIND DATE -------------------US 2002077292 A1 20020620 US 2001-966893 A2 20030410 WO 2002-US24194 PΙ 20010928 WO 2003029403 20020731 W: AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NO, NZ, OM, PH, PL, PT, RO, RU, SD, SE, SG, SI, SK, SL, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VN, YU, ZA, ZM, ZW RW: GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW, AM, AZ, BY, KG, KZ, MD, RU, TJ, TM, AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE, SK, TR, BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG AU 2002327407 A1 20030414 AU 2002-327407 20020731 US 2004126370 A1 20040701 US 2003-733501 20031211

- L11 ANSWER 9 OF 11 CAPLUS COPYRIGHT 2007 ACS on STN
- IN Amalfitano, Andrea; Chen, Yuan Tsong; Hu, Huimin
- TI Adenoviruses deleted in the IVa2, 100K and/or preterminal protein sequences
- SO PCT Int. Appl., 156 pp.

CODEN: PIXXD2 APPLICATION NO. PATENT NO. DATE KIND DATE -------------------PΙ WO 2000012740 A2 20000309 WO 1999-US19540 19990827 A3 WO 2000012740 20001123 AE, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, CA, CH, CN, CU, CZ, DE, DK, EE, ES, FI, GB, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MD, MG, MK, MN, MW, MX, NO, NZ, PL, PT, RO, RU, SD, SE, SG, SI, SK, SL, TJ, TM, TR, TT, UA, UG, US, UZ, VN, YU, ZA, ZW RW: GH, GM, KE, LS, MW, SD, SL, SZ, UG, ZW, AT, BE, CH, CY, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE, BF, BJ, CF, CG, CI, CM, GA, GN, GW, ML, MR, NE, SN, TD, TG CA 2340276 A1 20000309 CA 1999-2340276

ΑU	9956	942			<b>A1</b>	20000321	AU 1999-56942	19990827
ΑU	7646	86			B2	20030828		
ΕP	1108	049			A2	20010620	EP 1999-943952	19990827
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US	6328	958			В1	20011211	US 1999-384749	19990827
JP	2002	5280	56		Т	20020903	JP 2000-567725	19990827
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US	6797	265			B2	20040928		
AU	2003	2624	93		A1	20040108	AU 2003-262493	20031125
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- L11 ANSWER 10 OF 11 MEDLINE on STN DUPLICATE 1
- AU Schmidt B; Selmer T; Ingendoh A; von Figura K
- TI A novel amino acid modification in sulfatases that is defective in multiple sulfatase deficiency.
- SO Cell, (1995 Jul 28) Vol. 82, No. 2, pp. 271-8. Journal code: 0413066. ISSN: 0092-8674.
- L11 ANSWER 11 OF 11 CAPLUS COPYRIGHT 2007 ACS on STN DUPLICATE 2
- AU Litjens, Tom; Morris, C. Phillip; Gibson, Gary J.; Beckmann, Kerri R.; Hopwood, John J.
- TI Human N-acetylgalactosamine-4-sulphatase: protein maturation and isolation of genomic clones
- SO Biochemistry International (1991), 24(2), 209-15 CODEN: BIINDF; ISSN: 0158-5231

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## L11 ANSWER 9 OF 11 CAPLUS COPYRIGHT 2007 ACS on STN

The present invention provides deleted adenovirus vectors. The inventive adenovirus vectors carry one or more deletions in the IVa2, 100K, polymerase and/or preterminal protein sequences of the adenovirus genome. In the human adenovirus serotype 5 genomes, such deletions are at nucleotide positions 4830-5766, 24,990-25,687, and/or 7274-7991. adenoviruses may addnl. contain other deletions, mutations or other modifications as well. In particular preferred embodiments, the adenovirus genome is multiply deleted, i.e., carries 2 or more deletions therein. The deleted adenoviruses of the invention are "propagation-defective" in that the virus cannot replicate and produce new virions in the absence of complementing function(s). Preferred adenovirus vectors of the invention carry a heterologous nucleotide sequence encoding a protein or peptide associated with a metabolic disorder, more preferably a protein or peptide associated with a lysosomal or glycogen storage disease, most preferably, a lysosomal acid  $\alpha$ -glucosidase. The deleted adenovirus vectors advantageously have an increased carrying capacity for heterologous nucleotide sequences, demonstrate lower levels of viral protein expression, induce fewer host immune responses, and/or exhibit increased stability and prolonged transgene expression when introduced into target cells.

(FILE 'HOME' ENTERED AT 18:40:17 ON 17 MAY 2007) FILE 'MEDLINE, CAPLUS, BIOSIS, SCISEARCH, LIFESCI' ENTERED AT 18:40:34 ON 17 MAY 2007 11835 S LYSOSOMAL (4A) (DISEASE OR DISORDER) Ll 985 S (TREAT? OR INHIBIT? OR AMELIORAT? OR DECREAS? OR REDUC?) (8A) L L2147288 S GLUCOCEREBROSIDASE OR CERAMIDASE OR GALACTOCEREBROSIDASE OR S L3212 S L2 AND L3 L4121 DUP REM L4 (91 DUPLICATES REMOVED) L5 10097 S (GENE OR DNA OR CDNA OR POLYNUCLEOTIDE OR NUCLEIC (W) ACID) (8A) L6 L7 36 S L2 AND L6 L8 20 DUP REM L7 (16 DUPLICATES REMOVED) L9 7112 S L3(5A) (PROTEIN OR POLYPEPTIDE) 16 S L2 AND L9 L10 11 DUP REM L10 (5 DUPLICATES REMOVED) L11 3243202 S PROBLEMS OR DIFFICULT? OR SHORTCOME OR FAILURE OR PITFALL L12 60 S L2 AND L12 L13 L14 12 S L13 AND REVIEW L15 12 DUP REM L14 (0 DUPLICATES REMOVED) => d au ti so pi 1-12 l15 ANSWER 1 OF 12 CAPLUS COPYRIGHT 2007 ACS on STN L15 Wraith, J. E. UΑ Limitations of enzyme replacement therapy: current and future TI SO Journal of Inherited Metabolic Disease (2006), 29(2/3), 442-447 CODEN: JIMDDP; ISSN: 0141-8955 L15 ANSWER 2 OF 12 CAPLUS COPYRIGHT 2007 ACS on STN ΑU Pastores, Gregory M. ΤI Miglustat: substrate reduction therapy for lysosomal storage disorders associated with primary central nervous system involvement SO Recent Patents on CNS Drug Discovery (2006), 1(1), 77-82 CODEN: RPCDC9; ISSN: 1574-8898 L15 ANSWER 3 OF 12 CAPLUS COPYRIGHT 2007 ACS on STN Pastores, Gregory M.; Barnett, Natalie L. ΑU TICurrent and emerging therapies for the lysosomal storage disorders SO Expert Opinion on Emerging Drugs (2005), 10(4), 891-902 CODEN: EOEDA3 L15 ANSWER 4 OF 12 CAPLUS COPYRIGHT 2007 ACS on STN ΑU Kleta, Robert; Gahl, William A. TI Pharmacological treatment of nephropathic cystinosis with cysteamine SO Expert Opinion on Pharmacotherapy (2004), 5(11), 2255-2262 CODEN: EOPHF7; ISSN: 1465-6566 L15 ANSWER 5 OF 12 CAPLUS COPYRIGHT 2007 ACS on STN AU Pastores, G. M. Agalsidase alfa: Enzyme therapy for Anderson-Fabry disease TI SO Therapy (2004), 1(2), 203-211 CODEN: THERCR L15 ANSWER 6 OF 12 CAPLUS COPYRIGHT 2007 ACS on STN ΑU Nash, Robert ΤI Remedies from nature Chemistry World (2004), 1(7), 42-44 CODEN: CWHOBI; ISSN: 1473-7604

L15 ANSWER 7 OF 12 BIOSIS COPYRIGHT (c) 2007 The Thomson Corporation on STN AU Desnick, Robert J. [Reprint Author]; Brady, Roscoe; Barranger, John;

- Collins, Allan J.; Germain, Dominique P.; Goldman, Martin; Grabowski, Gregory; Packman, Seymour; Wilcox, William R.
- TI Fabry disease, an under-recognized multisystemic disorder: Expert recommendations for diagnosis, management, and enzyme replacement therapy.
- SO Annals of Internal Medicine, (18 February 2003) Vol. 138, No. 4, pp. 338-346. print.

  ISSN: 0003-4819 (ISSN print).
- L15 ANSWER 8 OF 12 CAPLUS COPYRIGHT 2007 ACS on STN
- AU Platt, F. M.; Jeyakumar, M.; Andersson, U.; Priestman, D. A.; Dwek, R. A.; Butters, T. D.; Cox, T. M.; Lachmann, R. H.; Hollak, C.; Aerts, J. M. F. G.; Van Weely, S.; Hrebicek, M.; Moyses, C.; Gow, I.; Elstein, D.; Zimran, A.
- TI Inhibition of substrate synthesis as a strategy for glycolipid lysosomal storage disease therapy
- SO Journal of Inherited Metabolic Disease (2001), 24(2), 275-290 CODEN: JIMDDP; ISSN: 0141-8955
- L15 ANSWER 9 OF 12 CAPLUS COPYRIGHT 2007 ACS on STN
- AU Maire, I.
- TI Is genotype determination useful in predicting the clinical phenotype in lysosomal storage diseases?
- SO Journal of Inherited Metabolic Disease (2001), 24(Suppl. 2), 57-61 CODEN: JIMDDP; ISSN: 0141-8955
- L15 ANSWER 10 OF 12 CAPLUS COPYRIGHT 2007 ACS on STN
- AU Asano, Naoki; Nash, Robert J.; Molyneux, Russell J.; Fleet, George W. J.
- TI Sugar-mimic glycosidase inhibitors: natural occurrence, biological activity and prospects for therapeutic application
- SO Tetrahedron: Asymmetry (2000), 11(8), 1645-1680 CODEN: TASYE3; ISSN: 0957-4166
- L15 ANSWER 11 OF 12 CAPLUS COPYRIGHT 2007 ACS on STN
- AU Asano, Naoki
- TI Alkaloidal sugar mimetics: Biological activities and therapeutic applications
- SO Journal of Enzyme Inhibition (2000), 15(3), 215-234 CODEN: ENINEG; ISSN: 8755-5093
- L15 ANSWER 12 OF 12 CAPLUS COPYRIGHT 2007 ACS on STN
- AU Brady, Roscoe O.
- TI Lysosomal storage diseases
- SO Pharmacology & Therapeutics (1983), 19(3), 327-36 CODEN: PHTHDT; ISSN: 0163-7258